Testimony of Rudolf Jaenisch, MD
Representing the American Society For Cell Biology
To the Science, Technology and Space Subcommittee
of the Senate Commerce Science and Transportation Committee
United States Senate

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Mr. Chairman and members of the Subcommittee, I am Rudolf Jaenisch and I am here today as a representative of the American Society For Cell Biology. The Society represents more than 10,000 basic biomedical researchers throughout the United States and the world, most of whom work in our Nation's leading research universities and institutes. It is my pleasure to appear before you today.

I am a founding Member of the Whitehead Institute and Professor of Biology at MIT. Before coming the Whitehead Institute I was the head of the Department of Tumor Virology at the Heinrich Pette Institute of the University of Hamburg in Germany. I am privileged to have helped establish the field of transgenic science. Transgenic science deals with the transfer of genes to create mouse models of human disease.

On March 28, I testified before the House Subcommittee on Oversight and Investigations at a hearing entitled "Issues Raised by Human Cloning Research." There I emphasized the scientific concerns of human cloning that have resulted from the problems encountered in animal cloning. Our experience with animal cloning allows us to predict with a high degree of confidence that few cloned humans will survive to birth and, of those, the majority will be abnormal. The most likely cause of abnormal clone development is faulty reprogramming of the genome. This may lead to abnormal gene expression of any of the 30,000 genes residing in the animal. Faulty reprogramming does not lead to chromosomal or genetic alterations of the genome, so methods that are used in routine prenatal screening to detect chromosomal or genetic abnormalities in a fetus cannot detect these reprogramming errors. There are no available methods now or in the foreseeable to future to assess whether the genome of cloned embryo has been correctly reprogrammed. The ASCB stated in 1998 its clear opposition to the cloning of a human being and remains a steadfast opponent today.

There is, however a critical distinction between the cloning of a human being – which is both morally questionable and scientifically dangerous – and the therapeutic cloning of cells for the purpose of developing tissue that may ultimately allow defective cells in people to be replaced by healthy cells. The Human Cloning Prohibition Act of 2001 prohibits the use of somatic cell nuclear transfer for the purposes of human cloning. This undoubtedly intended to prevent the cloning of a human being, but it also, perhaps inadvertently, would tragically limit biomedical research. Therapeutic cloning has the capability to turn human cells into specific tissue types, for example, to regenerate nerve cells and heart muscle cells, benefiting patients with Parkinson's, Alzheimer and heart disease. The potential benefits of therapeutic cell cloning are indisputable – the only uncertainty is when they will be realized.

Public reaction to animal cloning and the disreputable threats of human cloning are in grave danger of hindering critical research in embryonic stem cells for the repair of organs and tissues. Just over a year ago, a milestone in biomedical research was achieved when human embryonic stem lines were obtained by growing cells from the inner cell mass of early stage human embryos. Research work over the past 20 years using mouse embryonic stem cells has demonstrated the promise of these cells for basic research and potential disease therapy. ES cells by themselves cannot form a mouse, but they can differentiate into any of the cell types that comprise a mouse. Mouse ES cells have been used to elucidate many important aspects of normal mouse embryology and development, but, most important, mouse ES cells are currently being used in a variety of "proof of therapeutic principle" experiments in several animal models of human disease. For example, these cells appear to be able to produce neural progenitors that can repair spinal cord damage and reconstitute brain cells that produce the chemicals that control cognition, motion and sensory perception. If reproducible with human ES cells, this could lead to effective treatment of Parkinson's disease and Alzheimer's disease. Similarly, the production of healthy bone marrow cells to treat cancer and other hematopoietic diseases, and pancreatic cells to alleviate diabetes are all within reach, so long as well-intentioned efforts to prevent the cloning of human beings – living, talking, feeling, walking around human beings – do not have unintentionally interfere.

We may be on the cusp of a new era of medicine, one in which cell therapy could restore normal function to a variety of affected tissues using stem cells. To understand the need for rapid research progress with human pluripotent stem cells, one need look no further than many common, and often fatal, diseases such as cancer, heart disease and kidney disease. These diseases are treatable in whole or in part by tissue or organ transplants, but there are persistent and deadly problems of rejection and a woefully inadequate supply of suitable donor organs and tissues. In addition, the grim arithmetic of most organ transplants requires those who are seriously ill to wait for the tragic accidental death of another person so that they may live. Worse, for juvenile diabetes and many other diseases, there is not even a suitable transplantation therapy or other cure. Unless we use federal funds for all aspects of human pluripotent stem cell research new treatments for these conditions may be delayed by years, and many who might otherwise have been saved will surely die or endure needless suffering.

Cloning is an extremely complex area of biology in which the process itself is only now beginning to be understood. It is premature to ban a technique that is still in the process of evolving. At no point in our nation's history has Congress banned an area of scientific exploration or technology by federal legislation. We were at a similar crossroads 25 years ago with recombinant DNA technology, which indeed, as predicted, revolutionized science by spawning biotechnology and all of its medical and economic returns to this country. There is widespread support of the National Bioethics Advisory Commission's call for a voluntary international moratorium on human nuclear transfer for the purpose of creating a new human being. In addition, the Food and Drug Administration has specifically claimed that clinical research using cloning technology to create a human being is subject to FDA regulation under the Public Health Service Act and the Federal

Food, Drug and Cosmetic Act. The ASCB urges that if legislation is needed, it should specifically be concerned with the reproduction of a human being by nuclear transfer. At the same time, any legislation should not impede or interfere with existing and potential critical research fundamental to the prevention or cure of human disease. This research often includes the cloning of human and animal cell lines and DNA, but not whole human beings.

Thank you for the opportunity to provide testimony on this important issue.